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EMA/COMP/25354/2006 Rev.2
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

26 base single stranded phosphodiester DNA oligonucleotide for the treatment of pancreatic cancer

Please note that this product was withdrawn from the Community Register of designated orphan medicinal products in March 2011 on request of the sponsor.

On 16 February 2006, orphan designation (EU/3/06/352) was granted by the European Commission to Antisoma Research Ltd, United Kingdom, for 26 base single stranded phosphodiester DNA oligonucleotide for the treatment of pancreatic cancer.

What is pancreatic cancer?

Cancer that begins in the pancreas is called pancreatic cancer. The pancreas is a small organ that lies behind the stomach and in front of the spine. The pancreas has two main functions in the body. It makes a juice that helps to digest (break down) food. It also produces hormones, such as insulin, that helps to control blood sugar levels. About 95% of pancreatic cancers come from the cells that make the juice to digest. These cancers of the pancreas are called adenocarcinomas. Pancreatic cancer is life-threatening.

What is the estimated number of patients affected by the condition?

At the time of designation, pancreatic cancer affected less than 1 in 10,000 people in the European Union (EU)*. This is equivalent to a total of fewer than 46,000 people, and is below the threshold for orphan designation, which is 5 people in 10,000. This is based on the information provided by the sponsor and knowledge of the Committee for Orphan Medicinal Products (COMP).

What treatments are available?

The choice of the treatment of pancreatic cancer depends on several factors, including the stage of the disease. Treatments may include surgery, radiation therapy (using high-dose x-rays or other high

* Disclaimer: For the purpose of the designation, the number of patients affected by the condition is estimated and assessed on the basis of data from the European Union (EU 25), Norway, Iceland and Liechtenstein. This represents a population of 459,700,000 (Eurostat 2004).

energy rays to kill cancer cells), and chemotherapy (using drugs to kill cancer cells). There are anti-cancer drugs that have been authorised for treatment of pancreatic cancer.

26 base single stranded phosphodiester DNA oligonucleotide might be of potential significant benefit for the treatment of pancreatic cancer because it might act in a different way and thereby improve the long-term outcome of the patients. The assumption will have to be confirmed at the time of marketing authorisation. This will be necessary to maintain the orphan status.

How is this medicine expected to work?

Normal cells have proteins in their membranes (the outer sheet covering the whole cell and the thin envelop of the cell nucleus) that act as receptors. These receptors, after having interacted with different substances, then trigger some functions of the cell, like growth, migration, etc. These receptors are frequently altered in cancer cells, either in localisation, number or functional characteristics. 26 base single stranded phosphodiester DNA oligonucleotide seems to bind to a receptor that normally is present in the nuclear membrane only, but that in cancer cells is also present in the outer cell membrane. It is expected that when the product binds to the receptor on the tumour cell outer membrane, it might activate a series of events that eventually will kill the cancer cell by a process called apoptosis. By doing this, the product is expected to kill cancer cells.

What is the stage of development of this medicine?

The effects of 26 base single stranded phosphodiester DNA oligonucleotide were evaluated in experimental models.

26 base single stranded phosphodiester DNA oligonucleotide was not authorised anywhere worldwide for pancreatic cancer, at the time of submission. Orphan designation of 26 base single stranded phosphodiester DNA oligonucleotide was granted in the United States for pancreatic cancer.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 11 January 2006 recommending the granting of this designation.

Opinions on orphan medicinal product designations are based on the following three criteria:

- the seriousness of the condition;
- the existence of alternative methods of diagnosis, prevention or treatment;
- either the rarity of the condition (affecting not more than 5 in 10,000 people in the EU) or insufficient returns on investment.

Designated orphan medicinal products are products that are still under investigation and are considered for orphan designation on the basis of potential activity. An orphan designation is not a marketing authorisation. As a consequence, demonstration of quality, safety and efficacy is necessary before a product can be granted a marketing authorisation.

For more information

Sponsor's contact details:

Antisoma Research Ltd
Chiswick Park Building 5
566 Chiswick High Road
London W4 5YF
United Kingdom
Telephone: +44 203 249 2100
Telefax: +44 203 249 2101
E-mail: enquiries@antisoma.com

For contact details of patients' organisations whose activities are targeted at rare diseases see:

- [Orphanet](#), a database containing information on rare diseases which includes a directory of patients' organisations registered in Europe.
- [European Organisation for Rare Diseases \(EURORDIS\)](#), a non-governmental alliance of patient organisations and individuals active in the field of rare diseases.

Translations of the active ingredient and indication in all official EU languages¹, Norwegian and Icelandic

Language	Active Ingredient	Indication
English	26 base single stranded phosphodiester DNA oligonucleotide	Treatment of pancreatic cancer
Czech	Jednořetězcový fosfodiesterový DNA oligonukleotid o 26 bazích	Léčba karcinomu pankreatu
Danish	Enkeltstrenget phosphodiester-DNA-oligonukleotid med 26 baser	Behandling af pancreascancer
Dutch	26 base enkelstrandig fosfodiëster DNA oligonucleotide	Behandeling van pancreaskanker
Estonian	26 alusega üheaheelalise fosfodiester-DNA oligonukleotiid	Pankreasevähi ravi
Finnish	26-emäksinen yksijuosteinen fosfodiesteri-DNA-oligonukleotidi	Haimasyövän hoito
French	Oligonucléotide ADN à monobrin phosphodiester de 26 bases	Traitement du cancer pancréatique
German	26-Basen-, Einzelstrang-, Phosphodiester-, DNA-Oligonukleotid	Behandlung des Pankreaskarzinoms
Greek	Φωσφοδιεστεράση μονής ολιγονουκλεοτιδικής DNA αλύσου 26 βάσεων	Θεραπεία καρκίνου του παγκρέατος
Hungarian	26 bázisból álló, egyes szálú foszfodiészter DNS oligonukleotid	Hasnyálmirigyrák kezelése
Italian	Oligonucleotide fosfodiesteri del DNA a catena singola a 26 basi	Trattamento del cancro pancreatico
Latvian	26 bāzu vienpavediena fosfodiesterā DNS oligonukleotīds	Aizkuņģa dziedzera vēža ārstēšanai
Lithuanian	26 bazių DNR oligonukleotidas su viena fosfodiesterio jungtimi	Kasos vėžio gydymas
Polish	26 zasadowy oligonukleotyd fosfodiestrowy pojedynczego łańcucha DNA	Leczenie raka trzustki
Portuguese	Oligonucleótido de DNA fosfodiéster monocatenado com 26 bases	Tratamento do cancro do pâncreas
Slovak	Jednovláknový fosfodiester-oligonukleotid DNA zložený z 26 báz	Liečba rakoviny pankreasu
Slovenian	26 bazni enoverižni fosfodiester DNK oligonukleotid	Zdravljenje raka trebušne slinavke
Spanish	Oligonucleótido ADN fosfodiéster monocatenario compuesto por 26 bases	Tratamiento del cáncer de páncreas
Swedish	26-bas enkelsträngad fosfordiester-DNA-oligonukleotid	Behandling av pancreascancer
Norwegian	26-base enkelttrådig fosfodiester DNA-oligonukleotid	Behandling av pankreascancer
Icelandic	26 basa einþátta fosfótvíester DNA- ólígónúkleótíð	Meðferð briskrabbameins

¹ At the time of designation